Genome Editing of Autologous Hematopoietic Stem Cells to Treat Sickle Cell Disease

Grant Award Details

Genome Editing of Autologous Hematopoietic Stem Cells to Treat Sickle Cell Disease

Grant Type: Late Stage Preclinical Projects

Grant Number: CLIN1-10084

Project Objective: Genome Editing of Autologous Hematopoietic Stem Cells to Treat Sickle Cell Disease

Investigator:

Name:	Matthew Porteus
Institution:	Stanford University
Type:	PI

Disease Focus: Sickle Cell Disease, Blood Disorders

Award Value: $4,849,363

Status: Active

Grant Application Details

Application Title: Genome Editing of Autologous Hematopoietic Stem Cells to Treat Sickle Cell Disease

Public Abstract:

Therapeutic Candidate or Device

Autologous blood stem cells edited to correct the sickle cell disease mutation to be given back to the patient as an autologous stem cell transplant

Indication

Severe sickle cell disease

Therapeutic Mechanism

The mechanism of the proposed therapy for sickle cell disease is that the genetically engineered autologous HSCs (pathologic S allele corrected) will replace the endogenous HSCs using an autologous hematopoietic stem cell transplantation (HSCT). We will use ablative chemotherapy to eliminate the endogenous HSCs and create space for the genetically corrected HSCs. The genetically corrected HSCs will then produce red blood cells with Hgb A and should not sickle and cause disease.

Unmet Medical Need

Sickle cell disease patients have an average lifespan in the mid-40s with a life with frequent painful crisis. The only curative therapy is allogeneic HSCT but it has significant side effects and is only available to a small number of patients. Thus, there remains an unmet medical need.

Project Objective

Filing of IND application with the FDA

Major Proposed Activities

- Generate viral vector (AAV) that will be utilized by the blood stem cell to change the sickle cell disease mutation to a non-disease causing base
- Establish the reproducibility of the stem cell manufacturing process by repeating the clinical scale manufacturing process three times
- File an Investigator New Drug (IND) application with the FDA to get approval to start a phase I/II clinical trial

Statement of Benefit to California:

It is estimated that there ~5000 people living in California with sickle cell disease with ~100 new patients born in CA each year (CDC). The disease not only impacts the patients but also directly impacts families and communities. Thus, curing these patients would have tremendous personal benefit on the patients and their families. Moreover, the economic costs (both direct and indirect) are enormous and curing sickle cell disease would also provide a great economic benefit to the state.

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